



# 8th International Symposium on NBIA

**October 13-15, 2022**

**Website:** [>> more information <<](#)

**Lausanne, Switzerland**  
Mövenpick Hotel Lausanne

## Program committee:

Susan Hayflick, Oregon Health & Science University, Portland (US) | Thomas Klopstock, LMU Klinikum, Munich (DE) | Markus Nielbock, Hoffnungsbaum e.V., Würselen (DE)  
Agnès Rötig, Institut Imagine, Paris (FR) | Valeria Tiranti, IRCCS Istituto Neurologico Carlo Besta, Milan (IT)

## Local organizing committee:

Patrick Herren, Fatemeh Mollet, Esther Schärer, Olav Zilian, NBIA Suisse (patient advocacy association), Lausanne (CH)

## SCHEDULE

**THURSDAY**, October 13, 2022

13:00 Registration

14:00 **Welcome Address**  
Fatemeh Mollet, NBIA Suisse, Lausanne, Switzerland  
Patricia Wood, NBIA Disorders Association, El Cajon, USA  
Thomas Klopstock, LMU Klinikum, Munich, Germany

**SESSION 1** NBIA general Chair: Thomas Klopstock

14:15 **Key note lecture: molecular mechanisms of iron-sulfur protein biogenesis and its link to iron metabolism**  
Roland Lill, Marburg, Germany

15:00 **Epidemiology of NBIA disorders**  
Hana Kolářová, Prague, Czech Republic

15:20 **Unreported NBIA in known disease genes**  
Agnès Rötig, Paris, France

15:40 **Iron chelation in neurodegenerative disorders – state of the art and what it means for NBIA**  
David Devos, Lille, France

16:00 Break

SESSION 2	PKAN & CoPAN	Chair: Valeria Tiranti
16:30	<b>Massive iron accumulation in PKAN-derived neurons and astrocytes: light on the human pathological phenotype</b> Sonia Levi, Milan, Italy	
16:50	<b>Zebrafish models with inborn errors of CoA biosynthesis</b> Dario Finazzi, Brescia, Italy	
17:10	<b>Mitochondrial and Mitophagy in PKAN: insights from a <i>Drosophila</i> model</b> Zhihao Wu, Dallas, USA	
17:30	<b>Yeast as a model for functional analysis of mutations associated with PKAN disorder and for the identification of therapeutic molecules</b> Paola Goffrini, Parma, Italy	
17:50	<b>Modelling CoPAN in mice</b> Ivano Di Meo, Milan, Italy	
18:10	<b>Combination of a specific diet and the microbiome compensates lethality of a PKAN animal model</b> Hein Schepers, Groningen, the Netherlands	
18:30	End of the day	

## FRIDAY, October 14, 2022

08:30	<b>Update on the CoA-Z clinical trial</b> Penelope Hogarth, Portland, USA
08:50	<b>Optimization and efficacy of the clinical compound BBP-671 in a PKAN mouse model</b> Suzanne Jackowski, Memphis, USA
09:10	<b>The PanK Activator BBP-671 for PKAN: results from a phase 1 trial in healthy volunteers, and update on pivotal trial plans</b> Susanne A. Schneider, Munich, Germany
09:30	<b>Roundtable discussion on urgent needs and next steps in PKAN/CoPAN</b>
10:30	Break

SESSION 3	MPAN	Chair: Agnès Rötig
11:00	<b>Expanding our understanding of <i>C19orf12</i> function to develop therapeutic approaches for MPAN</b> Arcangela Iuso, Munich, Germany	
11:20	<b>Modelling <i>c19orf12</i> deficiency in zebrafish</b> Barbara Gnutti, Brescia, Italy	
11:40	<b>Roundtable discussion on urgent needs and next steps in MPAN</b>	
12:30	Break	

SESSION 4	BPAN	Chair: Susan Hayflick
13:30	<b>Is it all about autophagy? - WDR45's role in autophagy and beyond</b> Mario Mauthe, Groningen, the Netherlands	
13:50	<b>Towards an understanding of the molecular function of WDR45/WIPI4 in autophagy and BPAN</b> Tassula Proikas-Cezanne, Tübingen, Germany	
14:10	<b>Vulnerability of midbrain dopaminergic neurons in BPAN</b> Lena Burbulla, Munich, Germany	
14:30	<b>Characterization of a whole-body <i>Wdr45</i> knock-out, a mouse model for BPAN</b> Arcangela Iuso, Munich, Germany	
14:50	<b>Roundtable discussion on urgent needs and next steps in BPAN</b>	
15:40	Flash talks - selected poster presentations	
15:50	Poster Session & Coffee Break	
17:15	End of the day	

SESSION 5	PLAN/INAD & FAHN	Chair: Ody Sibon
09:00	<b>Towards precision therapies for NBIA</b> Manju Kurian, London, UK	
09:20	<b>PLA2G6-associated neurodegeneration – update</b> Manju Kurian, London, UK	
09:40	<b>Developing gene therapy for PLAN and moving towards clinical trials</b> Ahad Rahim, London, UK	
10:00	<b>Moving forward with FAHN research</b> Sunita Venkateswaran, Ottawa, Canada	
10:20	<b>Roundtable discussion on urgent needs and next steps in PLAN/INAD &amp; FAHN</b>	
11:00	Closing remarks Thomas Klopstock, LMU Klinikum, Munich, Germany	
11:15	End of the conference	

**Contact:**  
NBIA Suisse  
Fateme Mollet  
[Nbiasuisse@gmail.com](mailto:Nbiasuisse@gmail.com)